



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetate salt for the treatment of primary ciliary dyskinesia

On 27 February 2017, orphan designation (EU/3/17/1834) was granted by the European Commission to Polyphor UK Ltd, United Kingdom, for cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetate salt (also known as POL6014) for treatment of primary ciliary dyskinesia.

What is primary ciliary dyskinesia?

Primary ciliary dyskinesia is an inherited disease in which the cilia, hair-like structures which can be found in the lining of the airways, are defective. In the airways, cilia are important for sweeping out mucus and organisms that can cause infections and disease. Patients with primary ciliary dyskinesia are prone to recurring lung infections.

Although the main symptoms affect the lung and respiratory tract, patients can have problems with other organs including the ear and may also suffer from infertility. In a few patients with primary ciliary dyskinesia, organs in the chest and abdomen, such as the heart, are structurally abnormal or in the wrong position.

Primary ciliary dyskinesia is debilitating in the long-term term because of the recurring respiratory infections which can damage the lungs, and problems with heart and hearing and with infertility.

What is the estimated number of patients affected by the condition?

At the time of designation, primary ciliary dyskinesia affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 people^{*}, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

^{*}Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).



What treatments are available?

At the time of orphan designation, no suitable treatments were authorised for primary ciliary dyskinesia in the EU. Antibiotic treatments were widely used to treat recurring infections.

How is this medicine expected to work?

Patients with primary ciliary dyskinesia have high levels of neutrophil elastase, an enzyme released by white blood cells that is known to damage tissues when produced in excess.

When given by inhalation this medicine blocks the action of neutrophil elastase in the lung. By attaching to the sites through which neutrophil elastase exerts its activity, the medicine is expected to reduce or slow down the destruction of the lung tissue, thereby improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with primary ciliary dyskinesia had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for primary ciliary dyskinesia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 January 2017 recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetate salt	Treatment of primary ciliary dyskinesia
Bulgarian	Цикло[L-аланил-L-серил-L-изолевцил-L-пролил-L-пролил-L-глутаминил-L-лизил-L-тирозил-D-пролил-L-пролил-(2S)-2-аминодеканойл-L-алфа-глутамил-L-треонил] ацетат	Лечение на първична цилиарна дискинезия
Croatian	Ciklo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lizil-L-tirozil-D-prolil-L-prolil-(2S)-2-aminodekanoil-L-alfa-glutamil-L-treonil] acetatna sol	Liječenje primarne cilijarne diskinezije
Czech	Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetate salt	Léčba primární ciliární dyskineze
Danish	Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alfa-glutamyl-L-threonyl]acetat salt	Behandling af primær cilie dyskinesi
Dutch	Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetaatzout	Behandeling van primaire ciliaire dyskinesie
Estonian	Tsüklo[L-alanüül-L-serüül-L-isoleutsüül-L-prolüül-L-prolüül-L-glutaminüül-L-lüsüül-L-türosüül-D-prolüül-L-prolüül-(2S)-2-aminodekanoüül-L-alfa-glutamüül-L-treonüül]atsetaatsool	Primaarse tsiliaarse düskineesia ravi
Finnish	Syklo[L-alanyyli-L-seryyli-L-isoleussyli-L-prolyyli-L-prolyyli-L-glutaminyyli-L-lysyli-L-tyrosyyli-D-prolyyli-L-prolyyli-(2S)-2-aminodekanoyli-L-alfa-glutamyli-L-treonyyli]asetatisuola	Primaarisen siliaarisen dyskinesian hoito
French	Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodécanoyl-L-alpha-glutamyl-L-thréonyl], sel d'acétate	Traitement de la dyskinesie ciliaire primitive
German	Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminy-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetat salz	Behandlung der primären ciliären Dyskinesie
Greek	Κύκλο [L-αλανυλο-L-σερυλο-L-ισολευκυλο-L-προλυλο-L-προλυλο-L-γλουταμινυλο-L-λυσυλο-L-τυρόσυλο-D-προλυλο-L-προλυλο-(2S)-2-αμινοδεκανούλο-L-α-γλουταμυλο-L-θρεονυλο]οξικό άλας	Θεραπεία της πρωτοπαθούς δυσκινησίας των κροσσών
Hungarian	Ciklo[L-alanil-L-szeril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lizil-L-tirozil-D-prolil-L-prolil-(2S)-2-aminodekanoil-L-alfa-glutamil-L-treonil]acetát	Primer ciliaris dyskinesia kezelése

¹ At the time of designation

Language	Active ingredient	Indication
Italian	Ciclo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lisil-L-tyrosil-D-prolil-L-prolil-(2S)-2-aminodecanoil-L-alfa-glutamil-L-threonil]sale acetato	Trattamento della discinesia ciliare primaria
Latvian	Ciclo[L-alanil-L-seril-L-izoleicil-L-prolil-L-prolil-L-glutaminil-L-lizil-L-tirozil-D-prolil-L-prolil-(2S)-2-aminodekanoil-L-alfa-glutamil-L-treonil]acetāta sāls	Primārās ciliārās diskinēzijas ārstēšana
Lithuanian	Ciklo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lizil-L-tirozil-D-prolil-L-prolil-(2S)-2-aminodekanoil-L-alfa-glutamil-L-treonil]acetato druska	Pirminės ciliarinės diskinezijos gydymas
Maltese	Melġ ta' cyclo[L-alanyl-L-seryl-L-izoleucyl-L-prolyl-L-prolyl-L-glutaminyl-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alpha-glutamyl-L-threonyl]acetate	Kura ta' diskinesija ċiljari primarja
Polish	Cyklo[L-alanylo-L-serylo-L-izoleucylo-L-prolino-L-prolino-L-glutamylo-L-lizylo-L-tyrozynylo-D-prolino-L-prolino-(2S)-2-aminodekanoilo-L-alfa-glutamylo-L-treonylu]octan	Leczenie pierwotnej dyskinezy rzęsek
Portuguese	Ciclo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lisil-L-tirosil-D-prolil-L-prolil-(2S)-2-aminodecanoil-L-alfa-glutamil-L-threonil]acetate salt	Tratamento da discinesia ciliar primária
Romanian	Ciclo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lisil-L-tirosil-D-prolil-L-prolil-(2S)-2-aminodecanoil-L-alfa-glutamil-L-threonil]acetat	Tratamentul dischineziei ciliare primare
Slovak	Cyklo[L-alanyl-L-seryl-L-izoleucyl-L-prolyl-L-prolyl-L-glutaminyl-L-lyzyl-L-tyrozyl-D-prolyl-L-prolyl-(2S)-2-aminodekanoyl-L-alfa-glutamyl-L-treonyl]acetátová soľ	Liečba primárnej ciliárnej dyskinezie
Slovenian	Ciclo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lizil-L-tirosil-D-prolil-L-prolil-(2S)-2-aminodecanoil-L-alfa-glutamil-L-threonil]acetat	Zdravljenje primarne ciliarne diskinezije
Spanish	Acetato de sal de ciclo[L-alanil-L-seril-L-izoleucil-L-prolil-L-prolil-L-glutaminil-L-lisil-L-tirosil-D-prolil-L-prolil-(2S)-2-aminodecanoil-L-alfa-glutamil-L-treonil]	Tratamiento de Síndrome de disquinesia ciliar
Swedish	Cyklo[L-alanyl-L-seryl-L-izoleucyl-L-prolyl-L-prolyl-L-glutaminyl-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-alfa-glutamyl-L-threonyl]acetate salt	Behandling av primär ciliär dyskinesi
Norwegian	Syklo[L-alanyl-L-seryl-L-izoleucyl-L-prolyl-L-prolyl-L-glutaminyl-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodekanoyl-L-alfa-glutamyl-L-treonyl]acetatsalt	Behandling av primær ciliær dyskinesi
Icelandic	Cýcló[L-alanyl-L-serýl-L-ísóleucýl-L-prólyl-L-prólyl-L-glútamínýl-L-lýsýl-L-týrósyf-D-prólyl-L-prólyl-(2S)-2-amínódecanóyl-L-alfa-glútamýl-L-threónýl]acetat salt	Meðferð við meðfæddum bifhárágalla (Primary Ciliary Dyskinesia)